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PRESS RELEASE

EpiBlok is developing a gene therapy for epilepsy

During an epileptic seizure, groups of neurons suddenly fire all at once, leading to involuntary movements and sensations. Possibilities for helping those who suffer from epilepsy are limited. EpiBlok Therapeutics GmbH was recently founded by Charité and the Medical University of Innsbruck. The company is developing a type of gene therapy in which an adeno-associated virus transports the gene for the neuropeptide dynorphin into selected neurons of the affected brain region. The goal is the long-term suppression of seizures, by having the neurons produce a reserve supply of dynorphin that can be released when needed. Charité BIH Innovation, the joint technology transfer office of Charité – Universitätsmedizin Berlin and the Berlin Institute of Health at Charité (BIH), supported EpiBlok's founders in the process of patenting the underlying invention.

Neurologists refer to epilepsy as an "electrical storm in the brain." All of a sudden, groups of neurons fire all at once, thus causing involuntary movements and sensory impairment. About five percent of people have at least one such seizure in their lifetime. Focal epilepsy, in which seizures originate from a specific region of the brain, is difficult to treat with medication, which also has strong side effects that impair learning and memory. "There's nothing we can really do for many epileptics," says Prof. Regine Heilbronn, head of the Gene Therapy Lab at the Department of Neurology at Charité – Universitätsmedizin and a co-founder of EpiBlok. "Not even epilepsy surgery guarantees that seizures will not recur in the future. That's why we developed a novel therapeutic approach."

"Drug on demand" therapy

The novel approach developed by the team at the recently founded EpiBlok Therapeutics GmbH relies on a gene vector that is introduced directly into the epileptic focus and continually produces protective neuropeptides there. In focal epilepsy, there is often an insufficiency of a small protein, the neuropeptide dynorphin. Therefore, the scientists introduced the dynorphin gene into the affected neurons using a gene vector. The neurons then began producing and storing the dynorphin peptide. Prof. Christoph Schwarzer, a neuropharmacologist at the University of Innsbruck and a co-founder of EpiBlok, explains what's special about the therapy: "This is 'drug on demand' therapy. The neurons only release the stored peptide when it is needed, i.e., when the neurons are over-excited, like at the beginning of an epileptic seizure. Dynorphin calms the neurons, and the storm passes."

The scientists have already shown that this gene therapy is safe in mice, where it reliably suppresses epileptic seizures for several months after only one application. As vehicles for dynorphin, they use adeno associated virus (AVV) vectors, which have already been clinically approved for gene therapy for certain diseases. For preclinical trials of the AAV-based gene therapy for focal epilepsy, Regine

Heilbronn received a €3.3 million grant from the German Federal Ministry of Education and Research's GO-Bio program.

Prof. Christopher Baum, Chairman of the BIH Board of Directors and Chief Translational Research Officer of Charité, is also enthusiastic about the spin-off: "In order to make new developments in gene therapy available to patients suffering from insufficiently treatable diseases, we absolutely need the energy of scientists who are willing to found new companies. In Berlin, we are currently establishing an ecosystem for gene and cell therapies that will enhance conditions for start-ups. EpiBlok has come into being at the right place at the right time. This is a way to turn research into health."

The next step: clinical trials in humans

Regine Heilbronn, Christoph Schwarzer, and their team now want to use the company to take the next step into the clinical environment. "Our aim with EpiBlok Therapeutics GmbH is to produce the AAV vector in sufficient quantity and quality to undertake an initial clinical trial." Heilbronn and her team received assistance to found EpiBlok from the SPARK-BIH program. Dr. Tanja Rosenmund, head of the SPARK program, is pleased by their shared success: "The team is developing the first gene therapy to be supported with grant funding, coaching, mentoring, and networking from the SPARK program. SPARK's goal is to promote inventions from the life sciences so that patients can benefit from more new products and therapies. We are pleased that this highly innovative project is now being developed further at EpiBlok."

In April 2022, the Patents and Licensing Team at Charité BIH Innovation entered into an exclusive licensing agreement with EpiBlok to use the founders' invention, for which Charité has applied for a patent. Dr. Bettina Büttner, Technology Manager on the Patents and Licensing Team, comments: "Patents and exclusive licensing agreements are an important foundation for a spin-off that is pursuing cost-intensive drug development. They block competitors' imitations and secure exclusive marketing rights. EpiBlok is the first spin-off and Charité's first licensing partner to pursue gene therapy treatment. We are very excited about the future development and progress of this promising treatment for focal epilepsy."

More information about the SPARK-BIH program is available here.

More information about EpiBlok is available here.

About the Berlin Institute of Health at Charité (BIH)

The mission of the Berlin Institute of Health at Charité (BIH) is medical translation: transferring biomedical research findings into novel approaches to personalized prediction, prevention, diagnostics and therapies and, conversely, using clinical observations to develop new research ideas. The aim is to deliver relevant medical benefits to patients and the population at large. As the translational research unit within Charité, the BIH is also committed to establishing a comprehensive translational ecosystem – one that places emphasis on a system-wide understanding of health and disease and that promotes change in the biomedical translational research culture. The BIH was founded in 2013 and is funded 90 percent by the Federal Ministry of Education and Research (BMBF) and 10 percent by the State of Berlin. The founding institutions, Charité – Universitätsmedizin Berlin and Max Delbrück Center for Molecular Medicine in the Helmholtz Association (MDC), were independent member entities within the BIH until 2020. Since 2021 the BIH has been integrated into Charité as its so-called third pillar. The MDC is now the Privileged Partner of the BIH.

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